CARVYKTI® (ciltacabtagene autoleucel) CARTITUDE-1 study

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Overview[1-4]

CARTITUDE-1 (MMY2001) was a phase 1b/2, open-label, single-arm, multicenter study evaluating CARVYKTI in patients with RRMM who had previously received a PI, an immunomodulatory agent, and an anti-CD38 antibody.

The combined analysis from the phase 1b/2 of the CARTITUDE-1 study evaluated the safety and efficacy of CARVYKTI in 97 patients with RRMM.

Key eligibility criteria[4]

- Adult patients with progressive MM per IMWG criteria
- ECOG PS ≤1
- Measurable disease
- ≥3 prior therapies or were double refractory
- Prior PI, immunomodulatory drug, and anti-CD38 therapy

Study design[1,2]

Screening (28 days)

↓

Apheresis

↓

Bridging therapy (as needed)

↓

Lymphodepletion (day -5 to -3)
Cy (300 mg/m^2) + Flu (30 mg/m^2)

↓

CARVYKTI infusion (day 1)
Target: 0.75×10^6 (range, 0.5-1.0×10^6) CAR+ viable T cells/kg

↓

Post-infusion assessments
(day 1 to day 100)
Safety, efficacy, PK, PD, biomarker

↓

Post-treatment assessments
(day 101 up to the end of cohort)
Safety, efficacy, PK, PD, biomarker

↓

Follow-up
≥2 years after study drug infusion

Efficacy[2,3,5,6]

- Overall, 97 patients were treated with CARVYKTI, with a median follow-up of 33.4 months (range, 1.5-45.2).
- ORR was 97.9% (sCR, 82.5%; VGPR, 12.4%; PR, 3.1%).
- At a median follow-up of 27.7 months, 56/61 patients (91.8%) were MRD-negative at 10^{-5} sensitivity level and 39/52 patients (75.0%) were MRD-negative at 10^{-6} sensitivity level. At a median follow-up of 33.4 months, sustained MRD negativity for ≥12 months was reported in 26/49 patients. At 24 months post CARVYKTI infusion, 18 patients remained MRD negative with ≥CR.
- Median DOR was 33.9 months (95% CI, 25.5-NE).
- Median PFS was 34.9 months (95% CI, 25.2-NE) and median OS was NR.
- At the 33.4 month follow-up, an estimated 62.9% of patients were alive.

Safety[3,4,6]

- CRS occurred in 95% of patients, with grade 3/4 CRS occurring in 4.1% of patients and grade 5 CRS occurring in 1 patient. No new events of CRS have occurred since the median 12.4 month follow-up.
- At a median follow-up of 33.4 months (final analysis), total CAR-T cell neurotoxicities of any grade (including ICANS and other neurotoxicities) had occurred in 21.6% of patients (n=21), with grade 3/4 neurotoxicity occurring in 11.3% of patients (n=11). One treatment-related death was due to neurotoxicity. No new neurotoxicity events were reported since the 27.7-month follow-up.[3,6]
- The most common (≥20%) hematologic AEs of any grade were neutropenia (95.9%), anemia (81.4%), thrombocytopenia (79.4%), leukopenia (61.9%), and lymphopenia (53.6%).
- Overall, 26 SPMs were reported in 20 patients.
- A total of 35 deaths occurred.

Long-term follow-up[14,15]

- At a median follow-up of 61.3 months, median OS was 60.7 months (95% CI, 41.9-NE). A total of 32 patients (33%) remained alive and progression-free ≥5 years after treatment with CARVYKTI; of these, 31 patients (96.9%) had achieved a sCR.[14]
- Among the 32 PFS patients at ≥5 years, 12 patients in sCR were both MRD negative (at least 10^{-5} threshold) and imaging-negative at ≥5 years after CARVYTKI infusion. Of the remaining 20 patients, 12 patients were MRD-negative at the 10^{-6} threshold, 1 was MRD-positive, and the rest were unevaluable.[15]
- The safety profile of CARVYKTI remained consistent over long-term follow-up with earlier data cuts in 32 patients with ≥5 years of PFS.[14,15,16]
- In the overall population (N=97), 3 additional secondary primary malignancies and 9 grade ≥3 infections were reported. Of the 47 total deaths, 17 occurred without disease progression.[15]

Note: AE, adverse event; BCMA, B-cell maturation antigen; CAR, chimeric antigen receptor; CD, cluster of differentiation; CI, confidence interval; CR, complete response; CRS, cytokine release syndrome; Cy, cyclophosphamide; DOR, duration of response; ECOG PS, Eastern Cooperative Oncology Group performance status; Flu, fludarabine; ICANS, immune effector cell-associated neurotoxicity syndrome; IMWG, International Myeloma Working Group; ISS, International Staging System; LOT, lines of therapy; MM, multiple myeloma; MRD, minimal residual disease; NE, not estimable; NR, not reached; ORR, overall response rate; OS, overall survival; PD, pharmacodynamics; PFS, progression-free survival; PI, proteasome inhibitor; PK, pharmacokinetics; PR, partial response; RRMM, relapsed/refractory MM; sCR, stringent complete response; SPM, secondary primary malignancy; VGPR, very good partial response.

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CARTITUDE-1 (MMY2001; NCT035548207) was a phase 1b/2, open-label study evaluating the efficacy and safety of CARVYKTI in patients with RRMM after ≥3 prior LOTs including a PI, an immunomodulatory drug, and an anti-CD38 mAb. The combined analysis from phase 1b/2 portions of the CARTITUDE-1 study evaluated the safety and efficacy of CARVYKTI in 97 patients with RRMM.[1-4,8]

- Patients received a single infusion of CARVYKTI at a target dose of 0.75×10^6 CAR+ T cells/kg (target range $0.5\text{-}1.0\times10^6$) 5-7 days after the start of lymphodepletion/conditioning regimen (Cy 300 mg/m^2 and Flu 30 mg/m^2 daily for 3 days on days -5 to -3).[3]
- Retreatment with CARVYKTI was permitted within the same dose range in patients with documented progressive disease ≥6 months after CARVYKTI infusion, best response of at least minimal response, and no ongoing hematologic (grade ≥3) or nonhematologic (grade ≥2) toxicities.[3]
- To further evaluate long-term efficacy and safety, patients from the CARTITUDE-1 trial will continue to be followed for up to 15 years post-infusion in a separate study (CARTinue, MMY4002; ClinicalTrials.gov identifier: NCT05201781).[3]

Study design[1,2,3]

Key eligibility criteria

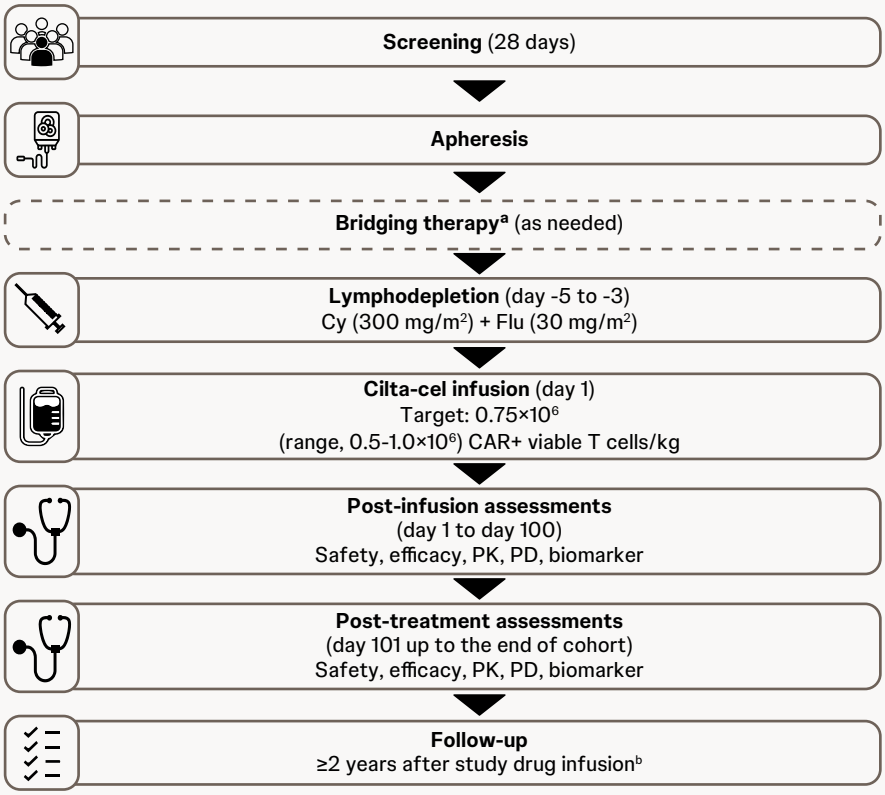
- Progressive MM per IMWG criteria
- ECOG PS ≤1
- Measurable disease
- ≥3 prior therapies or double refractory
- Prior PI, immunomodulatory drug, anti-CD38 therapy

Primary outcomes

- **Phase 1b:** characterize safety and confirm the RP2D
- **Phase 2:** evaluate efficacy by ORR

Key secondary outcomes

- sCR
- CR
- VGPR
- MRD negativity
- DOR
- PFS
- OS



[a]Treatment with previously used agent resulting in at least stable disease.
[b]Including a long-term, 15-year follow-up on a separate study.

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- Overall, of the 113 patients enrolled/apheresed, 101 were lymphodepleted and 97 were treated in the combined phase 1b/2 study.[3]
- Prior to lymphodepletion, a total of 12 patients discontinued treatment (progressive disease, n=2; withdrawal by patient, n=2; deaths, n=8). Prior to CARVYKTI infusion, 3 patients withdrew from treatment and 1 died.[3]
- No patient discontinued treatment due to manufacturing failure. Median turnaround time for CARVYKTI was 29 days (IQR, 28-33 days; defined as time from receipt to release of the product).[3,4]
- Patients received a median of 6 prior LOT (range, 3-18), 88% of patients were triple-class refractory, 42% penta-drug refractory, and 99% refractory to the last LOT.[3]
- The median administered dose was 0.71×10^6 CAR+ viable T cells/kg (range, $0.51\text{-}0.95\times10^6$).[3]

Select demographics and disease characteristics[1,3,4]

	Total (N=97)
Median age, years (range)	61.0 (56-68)
Male, n (%)	57 (59)
Black/African American, n (%)	17 (18)
Extramedullary plasmacytomas ≥1, n (%)	13 (13)
Bone marrow plasma cells ≥60%, n (%)	21 (22)
ECOG PS score, n (%)	
0	39 (40)
1	54 (56)
2	4 (4)
ISS stage, n (%)	
I	61 (63)
II	22 (23)
III	14 (14)
Median years since diagnosis (IQR)	5.9 (4.4-8.4)
Median prior LOT, n (IQR)	6 (3-18)
Prior LOT, n (%)	
3	17 (17.5)
4	16 (16.5)
≥5	64 (66.0)

Additional demographics and disease characteristics

Additional demographics and disease characteristics[1,3,4]

	Total (N=97)
High-risk cytogenetic profile, n (%)	23 (23.7)
del17p	19 (19.6)
t(14;16)	2 (2.1)
t(4;14)	3 (3.1)
Tumor BCMA expression ≥50%, n (%)	57 (92)[a]
Received bridging therapy, n	73
Prior stem-cell transplantation, n (%)	
Autologous	87 (90)
Allogenic	8 (8)
Triple-class exposed,[b] n (%)	97 (100)
Triple-class refractory	85 (88)
Penta-drug exposed,[c] n (%)	81 (84)
Penta-drug refractory	41 (42)
Refractory status, n (%)	
Carfilzomib	63 (65)
Pomalidomide	81 (84)
Daratumumab[d]	94 (97)
Refractory to last line of therapy, n (%)	96 (99)

[a]Denominator n=62, the number of evaluable samples; BCMA expression was detected in all evaluable samples.
[b]Those who received at least 1 PI, 1 immunomodulatory drug, and 1 anti-CD38 antibody.
[c]Those who received ≥2 PIs, ≥2 immunomodulatory drugs, and 1 anti-CD38 antibody.
[d]2 additional patients were refractory to other anti-CD38 antibodies.

BCMA, B-cell maturation antigen; CD, cluster of differentiation; PI, proteasome inhibitor.

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Efficacy response

- A total of 97 patients were evaluated for response.[2,3,8]
- Primary and updated analyses: median follow-up of 12.4 months, 18 months and 27.7 months.[2-5,8]

CARTITUDE-1: Efficacy response rates and outcomes[2-5,8]

Parameter	N=97		
	Median follow-up		
	12.4 Months	18 Months	27.7 Months
ORR,[a] % (95% CI)	97 (91.2-99.4)	97.9	97.9 (92.7-99.7)
Patients with a response,[b] n	94	-	95
sCR,[c] %	67	80.4	82.5 (73.4-89.4)[d]
VGPR,[c] %	26	14.4	12.4 (6.6-20.6)[d]
PR,[c] %	4	3.1	3.1 (0.6-8.8)[d]
Progressive disease,[c] %	1	-	1 (0-5.6)[d]
Median time to first response, months	1.0 (IQR, 0.9-1.0)	1.0 (range, 0.9-10.7)	1.0 (range, 0.9-10.7)
Median time to best response, months	2.6 (IQR, 1.0-6.1)	2.6 (range, 0.9-15.2)	2.6 (range, 0.9-17.8)
Median time to ≥CR, months	1.9 (IQR, 1.0-6.5)	2.6 (range, 0.9-15.2)	2.9 (range, 0.9-17.8)

[a]No patient had CR or stable disease.
[b]Sum of sCR, CR, VGPR, and PR.
[c]Data are in % unless otherwise specified.
[d]Data are in % (95% CI).

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Efficacy response

- At a median follow-up of 33.4 months (study closeout), median DOR was 33.9 months (95% CI, 25.5-NE).[6]

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PFS and OS

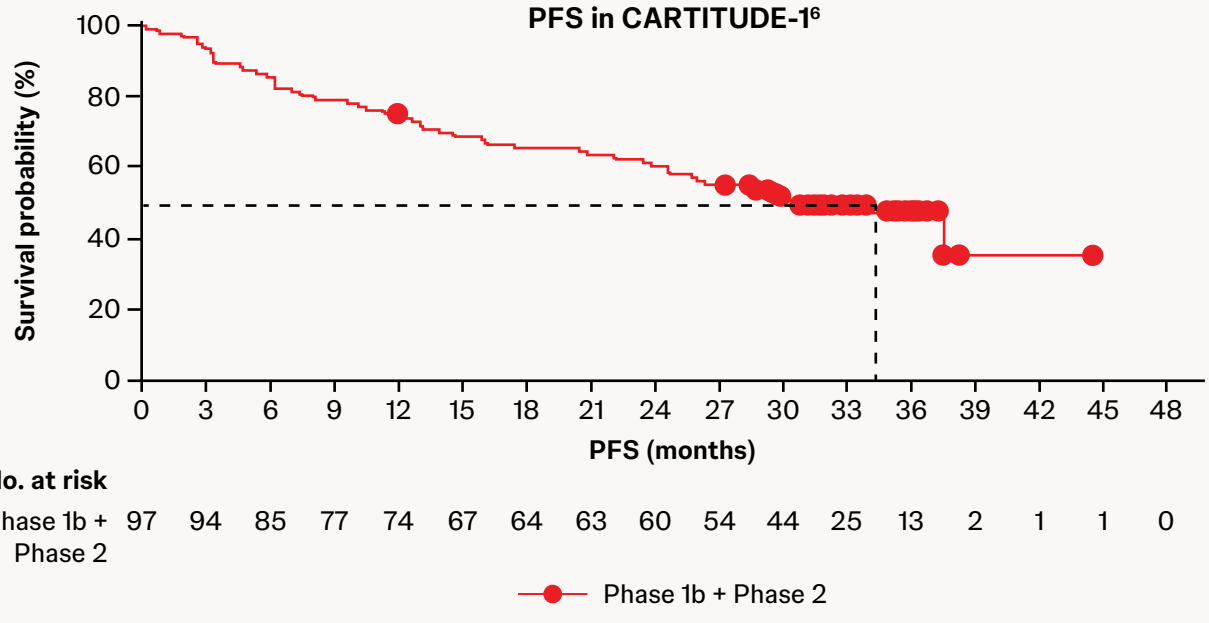
- PFS rates at 12 months, 18 months, and 27 months were 77.0% (95% CI, 66.0-84.3), 66.0% (95% CI, 54.9-75.0), and 54.9% (95% CI, 44.0-64.6), respectively.[3,8,10]
- OS rates at 12 months, 18 months, and 27 months were 89.0% (95% CI, 80.2-93.5), 80.9% (95% CI, 71.4-87.6), and 70.4% (95% CI, 60.1-78.6), respectively.[3,8,10]

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PFS and OS

- At a median follow-up of 33.4 months (study closeout), median PFS was 34.9 months (95% CI, 25.2-NE) and median OS was NR.[6]
- At the 33.4-month follow-up, an estimated 62.9% of patients were alive.[6]



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MRD negativity

- At a median follow-up of 27.7 months, of the 61 patients evaluable for overall MRD at a 10^{-5} sensitivity level, 56 patients (91.8%) were MRD-negative and of the 52 patients evaluable for overall MRD at a 10^{-6} sensitivity level, 39 patients (75.0%) were MRD-negative.[3]

MRD negativity at a median follow-up of 18 months and 27.7 months[2,3,5,8]

Parameter	N=97	
	Median follow-up	
	18 Months	27.7 Months
Evaluable patients for overall MRD, 10^{-5} sensitivity level,[a] n	61	61
Median time to MRD 10^{-5} negativity, months (range)	1 (0.8-7.7)	-
Overall MRD negativity at 10^{-5} sensitivity level, n (%)	(91.8)	56 (91.8)
MRD-negative sCR,[b] %, (95% CI)	-	44.3 (34.2-54.8)
≥CR and MRD negativity at 10^{-5} sensitivity level, %	89.4[c]	-
MRD negativity sustained ≥6 months,[d] %	-	68
MRD negativity sustained ≥12 months,[e] %	-	55
PFS rate in patients with MRD negativity sustained for ≥6 months, % (95% CI)	-	73.0 (52.1-85.9)
PFS rate in patients with MRD negativity sustained for ≥12 months, % (95% CI)	-	78.8 (51.5-91.8)
OS rate in patients with MRD negativity sustained for ≥6 months, % (95% CI)	-	93.5 (76.1-98.3)
OS rate in patients with MRD negativity sustained for ≥12 months, % (95% CI)	-	90.8 (67.7-97.6)
Evaluable patients for overall MRD, 10^{-6} sensitivity level, n	-	52
Overall MRD negativity at 10^{-6} sensitivity level, n (%)	-	39 (75.0)

[a]Evaluated by next-generation sequencing (clonoSEQ®).
[b]Intention-to-treat population.
[c]Denominator n=47; evaluable MRD sample within 3 months of achieving CR/sCR until death, progression, or subsequent therapy.
[d]34/50 patients with sufficient follow-up.
[e]24/44 patients with sufficient follow-up.

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- Baseline characteristics and treatment outcomes of patients with sustained MRD negativity among those treated with CARVYKTI in CARTITUDE-1 were analyzed at 6 and 12 months.[9]
  - MRD negativity (10^{-5} threshold) was assessed on bone marrow samples at baseline, on day 28, and at 6 months, 12 months, 18 months and 24 months after CARVYKTI infusion.
  - Sustained MRD negativity was defined in patients with 2 consecutive MRD-negative results after CARVYKTI infusion and before progression or subsequent therapy that were ≥6 months apart, with no MRD-positive results in between.
  - Patients with no MRD-negative results at any time point were defined as MRD-positive.

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- Of the 61 MRD evaluable patients, 56 patients (91.8%) achieved MRD negativity. The median follow-up duration was 28 months.[9]
- MRD negativity was sustained for <6 months in 22 patients, for 6-12 months in 10 patients, and for ≥12 months in 24 patients.[9]

Baseline characteristics in MRD subgroups[9]

Characteristic	MRD-negative for <6 months (n=22)	Sustained MRD-negative (N=34)	
		For 6-12 months (n=10)	For ≥12 months (n=24)
Median age, years (range)	59.5 (51-75)	66.0 (54-77)	63.0 (43-78)
Female, n (%)	8 (36.4)	6 (60.0)	13 (54.2)
Race, n (%)			
White	17 (77.3)	7 (70.0)	14 (58.3)
Black	4 (18.2)	2 (20.0)	4 (16.7)
Median time since diagnosis, years (range)	4.8 (1.6-16.3)	5.0 (1.6-8.1)	7.0 (2.5-18.2)
Plasmacytomas, n (%)	6 (27.3)	2 (20)	2 (8.3)
Extramedullary	4 (18.2)	2 (20)	1 (4.2)
Bone-based	2 (9.1)	0	1 (4.2)
High-risk cytogenetic profile, n (%)	6 (27.3)	2 (20)	6 (25.0)
ECOG PS at screening, n (%)			
0	10 (45.5)	4 (40.0)	12 (50.0)
1	10 (45.5)	6 (60.0)	12 (50.0)
2	2 (9.1)	0	0
ISS stage, n (%)			
I	15 (68.2)	5 (50.0)	18 (75.0)
II	6 (27.3)	2 (20.0)	4 (16.7)
III	1 (4.5)	3 (30.0)	2 (8.3)
Tumor BCMA expression ≥50%, n/N (%)	14/14 (100)	4/6 (66.7)	16/18 (88.9)
Previous stem cell transplant, n (%)			
Autologous	19 (86.4)	6 (60.0)	22 (91.7)
Allogeneic	1 (4.5)	0	2 (8.3)
Median number of prior LOT for MM (range)	5.0 (3-18)	4.5 (3-12)	5.5 (3-11)
Triple-class refractory,[a] n (%)	21 (95.5)	8 (80.0)	21 (87.5)
Penta-drug exposed,[b] n (%)	18 (81.8)	7 (70.0)	19 (79.2)
Penta-drug refractory,[b] n (%)	6 (27.3)	4 (40.0)	7 (29.2)
Refractory to last LOT, n (%)	22 (100)	10 (100)	24 (100)

[a]≥1 PI, ≥1 immunomodulatory drug, and 1 anti-CD38 antibody.
[b]≥2 PIs, ≥2 immunomodulatory drugs, and 1 anti-CD38 antibody.

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- 27.7-Month Follow-Up Analysis[9]:
  - All patients with sustained MRD negativity for ≥6 months achieved sCR.
  - Longer DOR and PFS were observed in patients with sustained MRD negativity.

Efficacy outcomes in MRD subgroups[9]

Parameter	MRD-negative for <6 months (n=22)	Sustained MRD-negative (n=34)	
		For 6-12 months (n=10)	For ≥12 months (n=24)
ORR, n (%)	21 (95)	10 (100)	24 (100)
sCR, %	55	100	100
VGPR, %	36	-	-
PR, %	5	-	-
Median DOR, months (95% CI)	10.3 (5.1-15.6)	NE (12.5-NE)	NE (NE-NE)
Median PFS, months 95% CI)	11.0 (5.4-16.6)	NE (13.4-NE)	NE (NE-NE)

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- 33.4-month final analysis[6]:
  - A total of 97 patients were evaluated for response at a median follow-up of 33.4 months, of whom 62 patients had samples evaluable for MRD at any time, and 49 patients had samples evaluable for 12-month sustained MRD.
    - Of the 49 evaluable patients, 26 patients achieved sustained MRD-negativity for ≥12 months
      - Of the 26 patients, 20 patients achieved sustained MRD-negativity with ≥CR.
  - At 24 months after administration of CARVYKTI, 18 patients remained MRD-negative with ≥CR.

PFS by CR and sustained MRD negativity[6]

Subgroups	mPFS (95% CI), months	30-Month PFS rate, %	36-Month PFS rate, %
All patients	34.9 (25.2-NE)	54.2	47.5
≥CR[a]	38.2 (34.9-NE)	66.8	59.8
12-month sustained MRD negativity[b]	NR (NE-NE)	74.9	NE
12-month sustained MRD negative ≥CR[a,b]	NR (NE-NE)	78.5	NE

[a]Patients had ≥CR at any time during the study, assessed by computerized algorithm.
[b]Patients who were MRD evaluable had a baseline clone identified, sufficient follow-up for assessment, and ≥2 MRD-negative assessments 12 months apart, with no MRD-positive samples in that interval.

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Patients retreated with CARVYKTI

- As of the data cutoff, 3 patients have been retreated with CARVYKTI and all were treated with the same dose as initial treatment.[3]

Response, CAR-T expansion, and ada status in patients retreated with CARVYKTI[3]

	Patient 1	Patient 2	Patient 3
Base response			
Initial treatment	sCR	sCR	VGPR
After retreatment	Progressive disease	Stable disease	Stable disease
CAR-T cell expansion after retreatment	None	None	None
ADA status			
Prior to retreatment	Positive	Negative	Negative
After retreatment	Data not available[a]	Negative	Negative

[a]One subject who was ADA positive after the initial treatment was pending additional data to confirm ADA status after retreatment.

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- Based on laboratory results, grade 3/4 thrombocytopenia occurred in 60 patients, of whom 20 patients (33.3%) had recovered to grade ≤2 by day 30 and 35 patients (58.3%) recovered by day 60. Grade 3/4 neutropenia was reported in 95 patients, of whom 66 patients (69.5%) had recovered to grade ≤2 by day 30 and 85 patients (89.5%) recovered by day 60. Grade 3/4 lymphopenia occurred in 96 patients, of whom 84 patients (87.5%) recovered to grade ≤2 by day 30 and 88 patients (91.7%) recovered by day 60.[3]
- The most common (≥5%) grade 3/4 nonhematologic AEs observed during the study were pneumonia (10.3%), hypophosphatemia (7.2%), increased gamma-glutamyl transferase (6.2%), hypertension (6.2%), fatigue (5.2%), and increased AST (5.2%).[3]
- Infections occurred in 56 patients (58%), with grade 3/4 infections observed in 19 patients (20%). The most common grade 3/4 infections were pneumonia (8%; n=8) and sepsis (4%; n=4). Upper respiratory tract infection was reported in 15 patients (16%); 1 patient had a grade 3 event.[4]
- At a median follow-up of 33.4 months (study closeout), a total of 26 SPMs were reported in 20 patients.[6]
  - At a median follow-up of 27.7 months, 16 patients had reported a total of 20 SPMs, all unrelated to CARVYKTI.[3]
    - Hematologic malignancies occurred in 9 patients (1 low-grade B-cell lymphoma, 6 MDS [1 case was fatal], W3 fatal AML [1 patient had both MDS and fatal AML]).[3]
    - A total of 6 non-melanoma skin cancers occurred; squamous cell carcinoma occurred in 4 patients (basal cell carcinoma also occurred in 1 of them) and 1 patient had basal cell carcinoma before CARVYKTI infusion.[3,5]
    - Malignant melanoma, adenocarcinoma, and myxofibrosarcoma occurred in 1 patient each.Prostate cancer occurred in 1 patient (the patient also had squamous cell carcinoma and AML reported above).[3]
  - Since the 27.7 months follow-up, 4 new patients developed SPMs, with 6 new cases including basal cell carcinoma (n=2) and 1 case each of MDS, B-cell lymphoma, melanoma, and prostate cancer.[6]
- A total of 35 deaths occurred during the study.[6]
  - Five new deaths unrelated to CARVYKTI were reported since the 27.7 months median follow-up (progressive disease, n=3; pneumonia, n=1; and sepsis, n=1).

Most common all grade AEs (≥20%)

Second primary malignancies

Deaths

Most common all grade AEs (≥20%)[3]

Event,[a] n (%)	N=97		
	All Grade	Grade 3/4	Grade 5
Hematologic AEs			
Neutropenia	93 (95.9)	92 (94.8)	0
Anemia	79 (81.4)	66 (68.0)	0
Thrombocytopenia	77 (79.4)	58 (59.8)	0
Leukopenia	60 (61.9)	59 (60.8)	0
Lymphopenia	52 (53.6)	49 (50.5)	0
Nonhematologic AEs			
Metabolism and nutrition disorders			
Hypocalcemia	31 (32.0)	3 (3.1)	0
Hypophosphatemia	30 (30.9)	7 (7.2)	0
Decreased appetite	28 (28.9)	1 (1.0)	0
Hypoalbuminemia	27 (27.8)	1 (1.0)	0
Hyponatremia	22 (22.7)	4 (4.1)	0
Hypokalemia	20 (20.6)	2 (2.1)	0
Gastrointestinal			
Diarrhea	29 (29.9)	1 (1.0)	0
Nausea	27 (27.8)	1 (1.0)	0
Constipation	22 (22.7)	0	0
Other			
Fatigue	36 (37.1)	5 (5.2)	0
Cough	34 (35.1)	0	0
AST increased	28 (28.9)	5 (5.2)	0
ALT increased	24 (24.7)	3 (3.1)	0
Chills	20 (20.6)	0	0
Pyrexia	20 (20.6)	0	0

[a]AEs were graded using NCI-CTCAE v5.0.

AE, adverse event; ALT, alanine aminotransferase; AST, aspartate aminotransferase; NCI-CTCAE, National Cancer Institute Common Terminology Criteria for Adverse Events.

Second primary malignancies[6]

SPM type	Patients with SPMs,[a] n
Hematologic malignancies	10
Myelodysplastic syndrome	7
AML	3
B-cell lymphoma	1
Cutaneous/noninvasive malignancies	8
Basal cell carcinoma	4
Squamous cell carcinoma	3
Malignant melanoma	2
Recurrent skin Squamous cell carcinoma	1
Squamous cell carcinoma of skin	1
Noncutaneous/invasive malignancies	4
Prostate cancer	2
Mucinous cystadenocarcinoma of the ovary	1
Myxofibrosarcoma	1

[a]A total of 20 patients had SPM; some had ≥1 SPM.

AML, acute myeloid leukemia; SPM, second primary malignancy.

Deaths[6]

	Total (N=97)	Time of death after CARVYKTI infusion (days)
Total	35	45-980
Due to progressive disease	17	253-980
AEs unrelated to treatment (n=12)		
Pneumonia	2	109, 887
AML[a]	3	418, 582, 718
Ascites[b]	1	445
MDS	1	803
Respiratory failure	3	733, 793, 829
Septic shock and/or sepsis	2	917, 945
AEs related to treatment (n=6)		
Septic shock and/or sepsis	2	45, 162
CRS/HLH	1	99
Lung abscess	1	119
Respiratory failure	1	121
Neurotoxicity	1	247

[a]One patient with AML also had MDS and a cytogenetic profile consistent with MDS (del20q [present before CARVYKTI infusion], loss of 5q); another patient who died from AML had both prostate cancer and squamous cell carcinoma of the scalp.
[b]Patient died from ascites unrelated to CARVYKTI as assessed by the investigator due to noncirrhotic portal fibrosis and nonalcoholic steatosis that was present for many years preceding the study.

AE, adverse event; AML, acute myelogenous leukemia; CRS, cytokine release syndrome; HLH, hemophagocytic lymphohistiocytosis; MDS, myelodysplastic syndrome.

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- CRS occurred in 95% of patients (92/97). Overall, 51% of patients (n=49) had grade 1 CRS and 39% of patients (n=38) had grade 2 CRS. Five CRS events were grade ≥3 (grade 3/4, n=4; grade 5, n=1).[1,4]
- Median time to CRS onset was 7 days (IQR, 5-8), with 89% of patients experiencing CRS onset at day 4 or later, and 74% at day 6 or later.[1,4]
- Median duration of CRS was 4 days (IQR, 3-6), and CRS resolved in 91 patients (99%) within 14 days of onset.[1,4]
- One death due to CRS/HLH was reported on day 99 after CARVYKTI infusion.[3]
- No new events of CRS (no changes in the incidence, time to onset, or duration) occurred since the 12.4-month median follow-up.[3]

CRS[3,4,10]

	Total (N=97)
Patient with CRS, n (%)	
Any grade[a]	92 (95)
Grade 1	49 (51)
Grade 2	28 (39)
Grade 3	3 (3)
Grade 4	1 (1)
Grade 5	1 (1)
Median time to onset of CRS, days (IQR)	7 (5-8)
CRS onset day 4 or later, n (%)	82 (89)
CRS onset day 6 or later, n (%)	68 (74)
Median duration of CRS, days (IQR)	4 (3-6)[b]
CRS resolved within 14 days of onset, n (%)	91 (99)
Supportive measures, n (%)	88 (91)
Tocilizumab	67 (69)
Corticosteroids	21 (22)
Anakinra	18 (19)
Oxygen	6 (6)
Vasopressor	4 (4)
Intubation/mechanical ventilation	1 (1)
Cyclophosphamide	1 (1)
Etanercept	1 (1)

[a]CRS was graded using Lee criteria in the phase 1b portion of the study and ASTCT criteria in phase 2; in this combined analysis, Lee et al. criteria were mapped to ASTCT criteria for patients in the phase 1b portion.
[b]One patient with a 97-day duration died due to CRS/HLH.

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- At a median follow-up of 33.4 months (range, 1.5-45.2; final analysis), total CAR-T cell neurotoxicity of any grade (including ICANS and other neurotoxicities) had occurred in 21 patients (21.6%).[3,4,6,11]
  - Grade 3/4 total CAR-T cell neurotoxicity occurred in 11 patients (11.3%).[3]
- ICANS of any grade occurred in 16 patients (17%) overall, with grade ≥3 events occurring in 2 patients (2%). One treatment-related death was considered related to neurotoxicity.[3,4,6,11]
  - ICANS resolved in all 16 patients. The median time to onset of ICANS was 8 days (IQR, 6-8), and the median duration was 4 days (IQR, 3-6.5).[4]
- No new neurotoxicity events have been reported since the 27.7-month median follow-up.[6]

Neurotoxicities[3,4,11]

	Total (N=97)
Total CAR-T cell neurotoxicities,[a] n (%)	
Any grade	21 (21.6)
Grade 3/4	11 (11.3)
Grade 5	1 (1.0)
ICANS, n (%)	
Any grade	16 (17)
Grade 3/4	2 (2)
Median time to onset of ICANS, days (IQR)	8 (6-8)
Median time to recovery from ICANS, days (IQR)	4 (3-6.5)

[a]Includes ICANS and other neurotoxicities. Neurotoxicity was graded using NCI-CTCAE v5.0 in phase 1b, and ICANS was graded by ASTCT criteria in phase 2. Note that ICANS and other neurotoxicities were not mutually exclusive, as 8% of patients had both ICANS and other neurotoxicity of any grade.

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- At a median follow-up of 18 months (range, 1.5-30.5), 12 patients (12.4%) had experienced other neurotoxicities of any grade (events not reported as ICANS [ie, having an onset after recovery from CRS and/or ICANS]). Other neurotoxicities of grade ≥3 were reported in 9 patients (9.3%). The median onset for these events was 26.5 days (range, 11-108) after CARVYKTI infusion, and median time to resolution was 70 days (range, 2-159). Among these 12 patients the following was observed[11]:
  - Seven patients experienced neurotoxicity manifesting as follows: facial paralysis (n =1); neurotoxicity (n=1); concentration impairment (n=1); diplopia (n=1); cranial nerve palsy (n=1); sensory loss, ataxia, peripheral motor neuropathy, and peripheral sensory neuropathy (all in 1 patient); altered mental status and nystagmus (both in 1 patient).
    - Five patients recovered from neurotoxicity and 2 patients died of other causes.
  - Five patients (5.2%) experienced MNTs, including a cluster of movement and cognitive and personality changes.
    - All 5 patients were male and had received prior bridging therapy; 3 of the 5 patients (60%) had high tumor burden at baseline.
    - Supportive measures for MNTs included the following: steroids, systemic chemotherapy (Cy), intrathecal chemotherapy (methotrexate, cytarabine), anakinra, dasatinib, siltuximab, and other agents (eg, carbidopa/levodopa, levetiracetam) with limited or no observed improvement in symptoms.
    - A sCR was achieved in 3 patients with MNTs and a VGPR was achieved in 2 patients with MNTs.
    - After CARVYKTI infusion, all 5 patients developed grade ≥2 CRS; 4 of these patients had ICANS, and 4 of these patients had high CAR-T cell expansion and persistence.
    - Analysis of clinical laboratory values showed higher ALC, absolute CD4+ T cells, and absolute CAR+ T cells on days 14, 21, and 28 after CARVYKTI infusion in patients with MNTs vs those patients without MNTs.
    - From days 14 to 100 after CARVYKTI infusion, the frequency of CAR+ T cells was higher in patients with MNTs than in patients with other neurotoxicities without MNTs or in patients with neither. After CARVYKTI infusion, peak levels of IL-6 and IFN-γ were also higher in patients with MNTs than in the other groups.
    - PK analysis of CAR transgene levels in the peripheral blood of patients with MNTs showed high CAR-T cell peak expansion and a trend of delayed T_{max} and high overall exposure (expansion and persistence). CSF examinations in 2 patients with MNTs with CSF samples available showed CAR+ T cells.
    - The CMC assessment of the manufacturing process of CAR-T vector batches for the 5 patients with MNTs showed no relevant deviations in the manufacturing process. There were no notable product characteristics that would have distinguished patients who had MNTs from those who did not experience events.

Characteristics of MNTs at a median follow-up of 18 months

Characteristics of MNTs at a median follow-up of 18 months[11]

Characteristic	Total (N=97)
Patients with MNT,[a] n (%)	5 (5.2)
Maximum toxicity grade, n (%)	
Grade 1	0
Grade 2	1 (1.0)
Grade 3	3 (3.1)
Grade 4	0
Grade 5	1 (1.0)
Median time to onset, days (range)	27.0 (14-108)
Outcome of neurotoxic event,[b] n (%)	
Recovered or resolved	0
Not recovered or not resolved	3 (3.1)[c]
Recovering or resolving	1 (1.0)
Fatal	1 (1.0)

[a]Events not reported as ICANS (i.e., onset after a period of recovery from CRS and ICANS).
[b]After CARVYKTI infusion, all 5 patients developed grade ≥2 CRS; 4 patients (80%) had ICANS, and 4 patients (80%) had high CAR T-cell expansion and persistence.
[c]Not recovered or not resolved at the time of data cutoff; 2 of these patients died due to other causes (1 due to septic shock and the other due to lung abscess).

CAR, chimeric antigen receptor; CRS, cytokine release syndrome; ICANS, immune effector cell-associated neurotoxicity syndrome; MNT, movement and neurocognitive treatment-emergent adverse event.

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- At a median follow-up of 27.7 months, 1 new case of signs/symptoms of parkinsonism (also termed MNTs) occurred for a total of 6 cases in the study.[3,5]
  - The patient (male) experienced grade 1 cognitive slowing, gait instability, neuropathy, and grade 3 tremor on day 914 after infusion.[3]
    - The patient had received 7 prior LOT, had a history of ongoing peripheral neuropathy at study entry, and had 2 risk factors for parkinsonism (grade 2 CRS and grade 3 ICANS).[3,5]
    - A lumbar puncture was performed which was negative for CAR-T cells.[3]
    - Symptoms did not improve with a short course of levodopa.
    - At data cutoff, and without further treatment, the patient was stable and functioning (able to work), with slight improvement in gait instability, and remained in sCR.
- Of the 6 patients with parkinsonism, 3 patients have died (2 from other underlying causes [sepsis and lung abscess] and 1 related to parkinsonism). Of the other 2 patients who were living at time of data cutoff, 1 patient recovered and the other was recovering (ongoing grade 2 symptoms).[3]

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- Patient management strategies to prevent or reduce parkinsonism were implemented in new and ongoing studies for patients with ≥2 risk factors.[2,3]
- Risk factors included high tumor burden (defined as bone marrow plasma cell ≥80%, serum M-spike ≥5 g/dL, serum free light chain ≥5000 mg/L), prior grade ≥2 CRS, prior ICANS, and high CAR T-cell expansion and persistence.[2]
- Preventative measures, monitoring, and management strategies included enhanced bridging therapy to reduce tumor burden, early and aggressive supportive care including steroids for any grade ICANS and tocilizumab for any-grade ICANS with concurrent CRS, handwriting assessments using a novel handwriting tool for early detection of neurotoxicity symptoms, and extended monitoring beyond the first 100 days after CARVYKTI infusion (up to 1 year).[7,11]
- Patient management strategies have reduced the incidence of parkinsonism from 6% in CARTITUDE-1 to <0.5% across other studies in the CARTITUDE program.[3]

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- CAR+ T cells revealed maximum peripheral expansion at a median of 12.7 days (range, 8.7-54.6 days).[4]
- Most patients who had 6-months follow-up had CARVYKTI CAR transgene concentration below the level of quantification (<50 CAR genes per mcg gDNA) in peripheral blood.[3,4]

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Subsequent antimyeloma therapies

- At a median follow-up of 27.7 months, 24 patients had received ≥1 subsequent antimyeloma therapy.[12]

Summary of subsequent antimyeloma therapies[12]

Common subsequent antimyeloma therapies (≥5%), n (%)	**Total (N=97)**
≥1 subsequent antimyeloma therapy	24 (24.7)
Dexamethasone	20 (20.6)
Cyclophosphamide	13 (13.4)
Selinexor	11 (11.3)
Belantamab mafodotin	10 (10.3)
Carfilzomib	9 (9.3)
Cisplatin	8 (8.2)
Etoposide	8 (8.2)
Bortezomib	7 (7.2)
Daratumumab	7 (7.2)
Talquetamab	5 (5.2)
Pomalidomide	5 (5.2)

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- A post hoc analysis of the CARTITUDE-1 study was conducted at a median follow-up of 61.3 months to report long-term follow-up results, including OS, ≥5-year progression-free outcomes, associated biomarkers, and safety.[14]
- Patients were offered enrollment in CARTinue (NCT05201781), a 15-year post-infusion follow-up study with patient evaluations per local standard of care, following completion of CARTITUDE-1.[14]

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- A total of 97 patients were infused with CARVYKTI and followed for a median duration of 61.3 months. Of these, 19 patients were lost to follow-up or died.
- Progressive disease occurred in 46 patients, of whom 33 were subsequently not evaluable for further follow-up due to withdrawal of consent (n=2), loss to follow-up (n=1), or death (n=30).
- Among the remaining patients, 32 remained progression-free for ≥5 years.
- At data cutoff, of 97 patients receiving CARVYKTI, 45 patients were alive and in long-term follow-up.

Baseline demographics and disease characteristics by ≥5-year progression- free status

Baseline demographics and disease characteristics by ≥5-year progression- free status[14]

Characteristic	All Infused (N=97)	≥5 Years alive and progression-free (n=32)	Progressive disease within 5 years (n=46)	≥5 Years progression-free vs Pd within 5 years, *P* Value[a]
Median age, years (range)	61.0 (56-68)	60.0 (43-78)	61.5 (47-77)	0.515
Male, (%)	58.8	53.1	58.7	0.650
Cytogenetics				
High-risk[b], n/N (%)	23/91 (25.3)	7/30 (23.3)[c]	12/45 (26.7)	0.793
>1 high-risk factors, n (%)	2/91 (2.2)	1 (3.3)	1 (2.2)	
Baseline tumor burden[d], n (%)				
High	16 (16.5)	2 (6.3)	8 (17.4)	0.305
Medium	22 (22.7)	7 (21.9)	11 (23.9)	
Low	59 (60.8)	23 (71.9)	27 (58.7)	
Bone marrow plasma cells, %, median (range)	20[e] (0-100)	5.0 (0.8-80.0)	24.0 (0.0-95.0)	0.053
Soluble BCMA, µg/L, median (range)	58.5[e] (3.7-1342.9)	36.0 (3.7-864.6)	58.5 (3.8-1342.9)	0.117
Extramedullary plasmacytomas, n (%)	13 (13.4)	4 (12.5)[f]	6 (13.0)	1.000
Previous LOT, median (range)	6.0 (3-18)	6.5 (3-14)	5.0 (3-18)	0.058
Triple-class[g] refractory, n (%)	85 (87.6)	29 (90.6)	39 (84.8)	0.513
Penta-drug[h] refractory, n (%)	41 (42.3)	15 (46.9)	15 (32.6)	0.241
Time from start of last LOT to progression on that line, months, median (range)	4.17 (0.0-48.6)[i]	3.98 (0.7-48.6)[j]	3.89 (0.7-21.5)[k]	-

[a]*P* values were calculated by using Fisher's test for categorical variables and Wilcoxon test for numerical variables without multiplicity adjustments.
[b]Either del17p, t(14;16), or t(4;14).
[c]Four patients had del17p, 2 patients had t(14;16), and the remaining 1 patient had a double hit of del17p and t(14;16).
[d]Low tumor burden was defined as meeting all following parameters (as applicable): bone marrow % plasma cell <50%, serum M-protein <3 g/dL, serum FLC <3,000 mg/L; high tumor burden was defined as meeting any of the following parameters: bone marrow % plasma cell ≥80%, serum M-protein ≥5 g/dL, serum FLC ≥5,000 mg/L; intermediate tumor burden did not fit either criteria of high or low tumor burden.
[e]n=96
[f]Extramedullary disease denotes soft tissue plasmacytoma that was not contiguous with bone.
[g]≥1 PI, ≥immunomodulatory drugs, and 1 anti-CD38 antibody.
[h]≥2 PIs, ≥2 immunomodulatory drugs, and 1 anti-CD38 antibody.
[i]n=90.
[j]n=29
[k]n=42

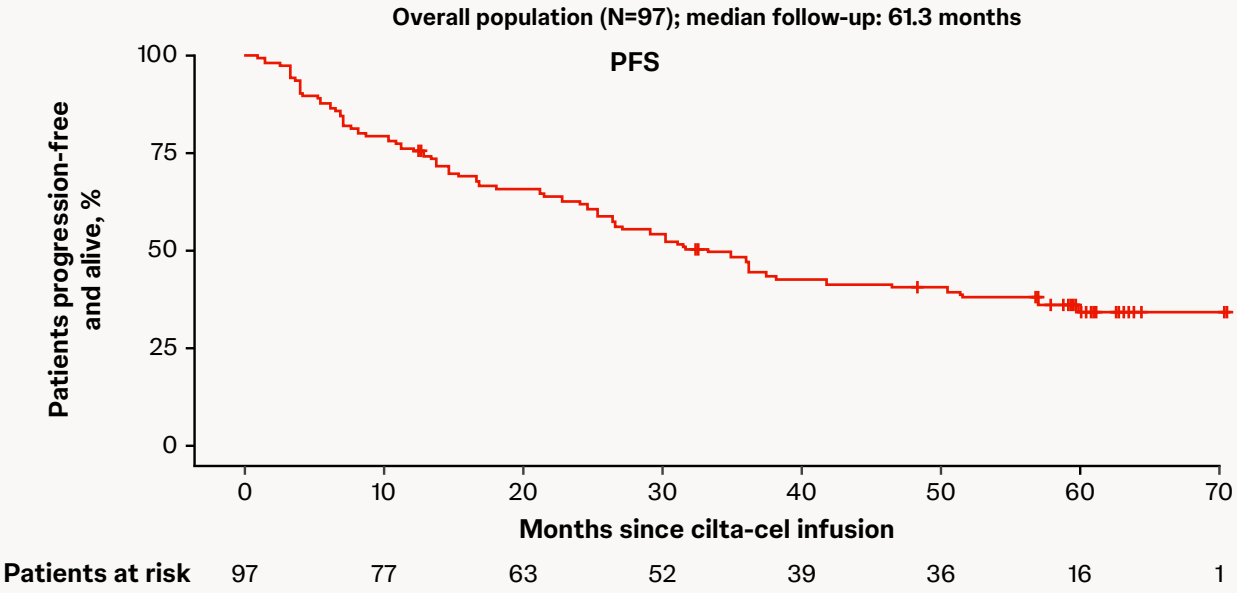
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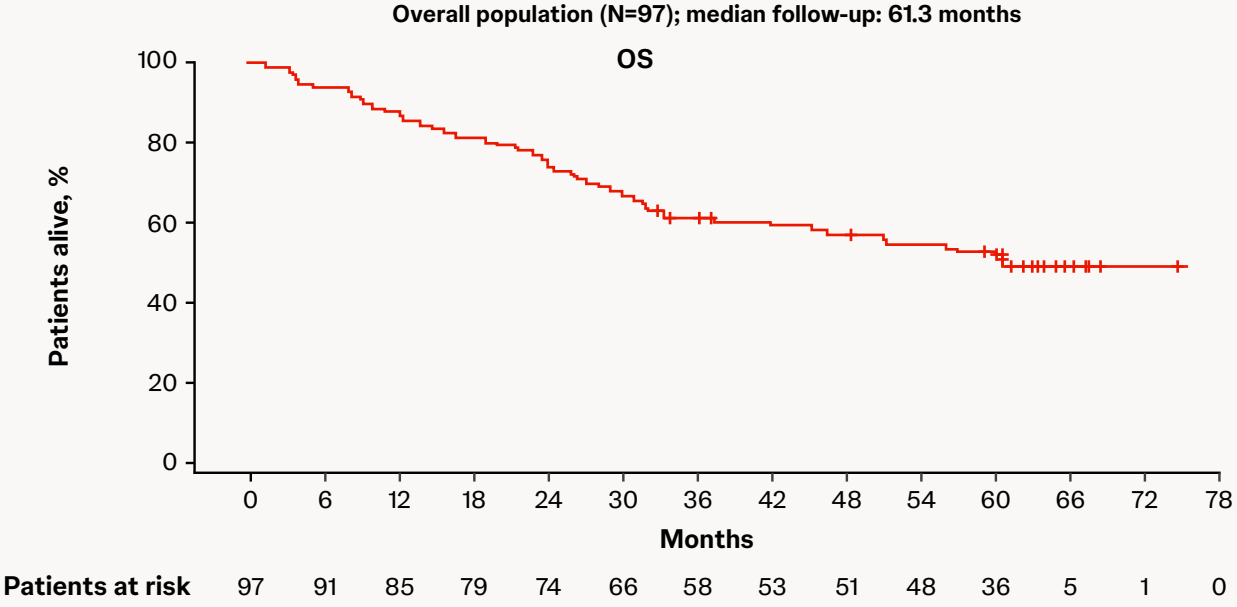
- Patients were followed up for a median period of 61.3 months.
- The median OS was 60.7 months (95% CI, 41.9-NE).[14]
- A total of 32/97 patients (33%) remained alive and progression-free without requiring any additional antimyeloma therapy ≥5 years after treatment with CARVYKTI; of these, 31 patients (96.9%) had achieved a sCR.[14]
- Among the 32 progression-free patients at ≥5 years, 12 patients in sCR underwent serial MRD and PET-tomography-computed assessments at a single center. All 12 of these patients were both MRD negative (at least 10^{-5} threshold) and imaging-negative at ≥5 years after CARVYTKI infusion. Of the remaining 20 patients, 12 patients were MRD-negative at the 10^{-6} threshold, 1 patient was MRD-positive, and the rest were unevaluable (5 lacked identifiable clones, 1 failed QC, and 1 was indeterminate) during the course of CARTITUDE-1.[15]
- Long-term disease control was associated with a higher E:T ratio (*P*=0.008) for most analyzed CAR-positive T-cell subsets with higher CAR+CD4^{+} central memory T-cell subsets and CAR+ T cells. Progression-free patients also had a lower neutrophil-to-leukocyte ratio (*P*=0.03), higher T cell-to-neutrophil ratio (*P*=0.05) at the time of apheresis, more CAR-positive naïve T cells in the product (*P*=0.003), higher CAR+ T-cell peak expansion (*P*=0.028), higher pre-infusion hemoglobin (*P*=0.001), higher platelet levels (*P*=0.049), and lower ferritin levels (*P*=0.158).[14,16]

Progression free survival and overall survival

Progression free survival[16]



Overall survival[16]



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- The safety profile of CARVYKTI remained consistent with earlier data cuts[3,4,6] in 32 patients with ≥5 years of PFS.[14,15,16]
  - No new cases of parkinsonism or cranial nerve palsy were observed. However, 2 secondary primary malignancies (solid tumors), 2 neurologic AEs, and 4 additional grade ≥3 infections (not related to CARVYKTI) were reported.
- In the overall population (N=97), at a 61.3-month follow-up, 3 additional cases of secondary primary malignancies (acute myeloid leukemia, lung adenocarcinoma, and anal squamous cell carcinoma) were reported; 9 patients had grade ≥3 infections. No new cases of parkinsonism or cranial nerve palsies were reported.[15]
- Among the 47 total deaths in CARVYKTI-treated patients overall, 17 occurred without progressive disease.[15]
  - Seven deaths within the first 12 months (infections, n=4; hemophagocytic lymphohistiocytosis, respiratory failure, and neurotoxicity, n=1 each)
  - Two deaths between 12 and 24 months (acute myeloid leukemia and ascites, n=1 each)
  - Six deaths between 24 and 36 months (infections, n=3; myelodysplastic syndrome, myocardial infarction, and respiratory failure, n=1 each)
  - Two deaths between 36 and 60 months (infection and acute myeloid leukemia, n=1 each).

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- The efficacy and safety of CARVYKTI were evaluated in key subgroups of patients in the CARTITUDE-1 study.[3,7]
- An analysis was conducted at a median follow-up of 21.7 months and 27.7 months in 97 patients in the following subgroups: age ≥65 years, Black/African American, 3 and ≥4 prior LOT, triple-class refractory, penta-drug refractory, cytogenetic risk status (including ≥1 chromosomal abnormality: del17p, t(4;14), and/or t(14;16)), ISS stage III disease at baseline, bone marrow plasma cell percentage at baseline (≤30%, >30 to <60%, and ≥60%), tumor BCMA expression at baseline (<median, ≥median), and presence of soft-tissue plasmacytomas (bone-based and extramedullary disease).[3,7]

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- Baseline cytogenetic data was available in 94% of patients (n=91); 24% of patients (n=23) had a high-risk cytogenetic profile based on the presence of ≥1 chromosomal abnormality including del17p (20%), t(4;14) (3%), and/or t(14;16) (2%).[3,7]
- A total of 96 patients had evaluable bone marrow biopsy and/or aspirate samples and 22% of patients (n=21) had high disease burden (≥60% plasma cells).[3,7]
- At baseline, plasmacytomas were detected in 20% of patients (n=19); of these, 13% (n=13) were EMD and 6% (n=6) were bone-based plasmacytomas.[3,7]

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- At data cutoff, 97 patients were evaluated for the efficacy and safety of CARVYKTI in key subgroups of patients with a median follow-up of 21.7 months (range, 1.5-33.9) and 27.7 months.[3,7]
- Most patients in high-risk subgroups responded (ORR range, 95.1%-100%) including those with 3 prior LOT (100%), high-risk cytogenetics (100%), high tumor burden (≥60% bone marrow plasma cells; 95.2%), and baseline plasmacytomas (100%).[3,7]
- MRD negativity rates at the 10^{-5} sensitivity were 80%-100% in evaluable patients across all subgroups.[3,5]
- DOR, PFS and/or OS were shorter in subgroups with high-risk cytogenetics, ISS stage III, high tumor burden and presence of plasmacytomas.[3,7]
  - A shorter DOR was observed in patients with 30%-60% bone marrow plasma cells.[3]
  - A reduced OS was observed in Black/African American patients.[3]

Select efficacy outcomes at a median follow-up of 27.7 months (subgroup analysis)[3]

	Patients, n (%)	ORR, % (95% CI)	Median DOR, months (95% CI)	MRD 10^{-5} negativity,[a] n (%)	Median PFS, (95% CI)	27-Month PFS, % (95% CI)	27-Month OS, % (95% CI)
Overall	97 (100)	97.9 (92.7-99.7)	NE (23.3-NE)	56/61 (91.8)	NE (24.5-NE)	54.9 (44.0-64.6)	70.4 (60.1-78.6)
≥65 years[b]	35 (36)	97.1 (85.1-99.9)	NE (24.3-NE)	21 (91.3)	NE (25.2-NE)	55.9 (36.2-71.7)	70.9 (52.6-83.2)
Black/African American	17 (18)	100.0 (80.5-100)	NE (6.8-NE)	10 (83.3)	NE (7.7-NE)	51.8 (26.2-72.4)	58.8 (32.5-77.8)
3 prior LOT	17 (18)	100.0 (80.5-100)	NE (12.9-NE)	8 (80.0)	NE (13.8-NE)	56.7 (30.0-76.6)	76.5 (48.8-90.4)
≥4 prior LOT	80 (82)	97.5 (91.3-99.7)	NE (20.2-NE)	48 (94.1)	30.1 (24.5-NE)	54.0 (41.7-64.8)	69.0 (57.3-78.1)
Triple-class refractory	85 (88)	97.6 (91.8-99.7)	NE (24.3-NE)	50 (92.6)	NE (25.2-NE)	55.6 (43.8-65.9)	69.7 (58.4-78.5)
Penta-drug refractory	41 (42)	95.1 (83.5-99.4)	NE (NE-NE)	17 (85.0)	NE (25.3-NE)	61.6 (44.0-75.1)	66.8 (49.3-79.4)
ISS stage III at baseline	14 (14)	100.0 (76.8-100)	14.1 (5.1-NE)	6 (100.0)	15.0 (6.1-NE)	34.3 (11.6-58.7)	50.0 (22.9-72.2)

[a]In MRD-evaluable patients; MRD was assessed in evaluable samples at 10^{-5} threshold by next-generation sequencing (clonoSEQ®) in all treated patients at day 28, and at 6, 12, 18, and 24 months regardless of the status of disease measured in blood or urine. Only MRD assessments (10^{-5} testing threshold) within 3 months of achieving CR/sCR until death/progression/subsequent therapy (exclusive) are considered. [b]There were 8 patients aged ≥75 years. No difference was observed in ORR between patients aged ≥75 years and with other age subgroups.

Additional efficacy outcomes at a median follow-up of 27.7 Months (subgroup analysis)

Additional efficacy outcomes at a median follow-up of 27.7 months (subgroup analysis)[3]

	Patients, n (%)	ORR, % (95% CI)	Median DOR, months (95% CI)	MRD 10^{-5} negativity,[a] n (%)	Median PFS, (95% CI)	27-Month PFS, % (95% CI)	27-Month OS, % (95% CI)
Cytogenetic risk							
Standard risk	68 (70)	97.1 (89.8-99.6)	NE (24.4-NE)	40 (95.2)	NE (25.3-NE)	57.8 (44.3-69.1)	72.9 (60.4-82.0)
High risk	23 (24)	100.0 (85.2-100)	20.2 (9.4-NE)	14 (82.4)	21.1 (10.8-NE)	43.5 (23.3-62.1)	64.6 (41.4-80.5)
Baseline bone marrow plasma cells							
≤30%	58 (60)	98.3 (90.8-100)	NE (25.7-NE)	28 (96.6)	NE (30.1-NE)	64.3 (50.0-75.5)	75.7 (62.4-84.8)
>30 to <60%	17 (18)	100.0 (80.5-100)	24.4 (15.9-NE)	14 (87.5)	25.3 (16.8-NE)	35.3 (11.6-60.5)	86.3 (54.7-96.5)
≥60%	21 (22)	95.2 (76.2-99.9)	23.1 (5.5-NE)	14 (87.5)	24.1 (6.5-NE)	45.9 (23.6-65.6)	45.9 (23.6-65.6)
Baseline tumor BCMA expression							
≥Median (80%)	31 (32)	96.8 (83.3-99.9)	NE (21.8-NE)	16 (94.1)	NE (26.4-NE)	65.8 (45.1-80.2)	75.9 (55.6-87.9)
<Median (80%)	31 (32)	100.0 (88.8-100)	NE (17.1-NE)	22 (95.7)	NE (18.0-NE)	54.8 (36.0-70.3)	71.0 (51.6-83.7)
Presence of baseline plasmacytomas[b]	19 (20)	100.0 (82.4-100)	12.9 (3.5-NE)	10 (90.9)	13.8 (5.3-NE)	47.4 (24.4-67.3)	52.1 (28.0-71.6)

[a]In MRD-evaluable patients; MRD was assessed in evaluable samples at 10^{-5} threshold by next-generation sequencing (clonoSEQ®) in all treated patients at day 28, and at 6, 12, 18, and 24 months regardless of the status of disease measured in blood or urine. Only MRD assessments (10^{-5} testing threshold) within 3 months of achieving CR/sCR until death/progression/subsequent therapy (exclusive) are considered.
[b]Includes bone-based and extramedullary plasmacytomas. 14 (87.5)

BCMA, B-cell maturation antigen; CI, confidence interval; CR, complete response; DOR, duration of response; MRD, minimal residual disease; NE, not estimable; ORR, overall response rate; OS, overall survival; PFS, progression-free survival; Pts, patients; sCR, stringent complete response.

CARVYKTI® (ciltacabtagene autoleucel) CARTITUDE-1 study

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- Incidence of CRS, ICANS, and other CAR-T cell neurotoxicities (events not reported as ICANS) in subgroups was consistent with the overall population.[7]
- No new safety signals were identified at longer median follow-up.[7]

CARVYKTI® (ciltacabtagene autoleucel) CARTITUDE-1 study

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- A retrospective analysis of efficacy and safety outcomes for patients with RRMM who had undergone alloSCT before receiving CARVYKTI in CARTITUDE-1 was conducted.[13]
- Key exclusion criteria in CARTITUDE-1 included having undergone alloSCT ≤6 months before apheresis. Patients who received alloSCT must have been off all immunosuppressive medications for 6 weeks without signs of GVHD.[13]

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- Of the 97 patients who received CARVYKTI infusion in CARTITUDE-1, 7 patients had previously received an alloSCT.[13]
  - These patients had received a median of 9 prior LOT (range, 6-14).
  - No patients received alloSCT as a component of the last LOT before enrollment.
  - All 7 patients had prior exposure to alkylating agents, with 5 patients having undergone radiotherapy.
  - All patients were refractory to bortezomib, lenalidomide, pomalidomide, and daratumumab; 5 patients were penta-drug refractory, comprising at least 2 PIs, 2 immunomodulatory drugs, and an anti-CD38 antibody.
- In addition to their prior alloSCT, all patients had previously undergone ≥1 autoSCT; 1 patient had received 2 prior autoSCTs.[13]

Baseline characteristics in patients with prior alloSCT

Baseline characteristics in patients with prior alloSCT[13]

Characteristic	Patients with prior alloSCT (n=7)
Median age, years (range)	60.0 (43-67)
Sex, n (%)	
Male	6(85.7)
Female	1 (14.3)
Race, n (%)	
Black or African American	1 (14.3)
White	5 (71.4)
Not Reported	1 (14.3)
Ethnicity, n (%)	
Not Hispanic or Latino	5 (71.4)
Not reported	2 (28.6)
Median time since diagnosis, years (range)	7.1 (5.4-10.3)
ECOG PS, n (%)	
0	5 (71.4)
1	2 (28.6)
ISS stage, n (%)	
I	4 (57.1)
II	1 (14.3)
III	2 (28.6)
Bone marrow plasma cells,[a] n (%)	
≤30%	6 (85.7)
>30% to <60%	0 (0)
≤60%	1 (14.3)
Cytogenetics, n (%)	
Standard risk	3 (42.9)
High risk	2 (28.6)
Del17p	2 (28.6)
Unknown	2 (28.6)
Tumor BCMA expression ≥50%, n (%)	
Yes	5 (71.4)
Not available	2 (28.6)
Median number of prior therapy, n (range)	9 (6-14)
Median time since alloSCT, years (range)	5.1 (2.7-6.2)

[a]Maximum value from bone marrow biopsy and bone marrow aspirate is selected if both the results are available; 1 subject with nonevaluable bone marrow biopsy/aspirate.

alloSCT, allogeneic stem cell transplant; BCMA, B-cell maturation antigen; ECOG PS, Eastern Cooperative Oncology Group performance status; ISS, International Staging System..

CARVYKTI® (ciltacabtagene autoleucel) CARTITUDE-1 study

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- At a median follow-up of 27.7 months (data cutoff, January 11, 2022), the ORR was 85.7% (n=6), sCR was 71.4% (n=5), and VGPR was 14.3% (n=1).[13]
- Due to the lack of measurable disease by any criteria at baseline, one patient with a prior alloSCT was designated as NE by the IRC and remained NE throughout the study assessment.[13]
- Median time to first response was 0.95 months (range, 0.9-1.0), median time to best response was 5.55 months (range, 1.0-17.1), and median DOR was NE for the 6 responders.[13]
- Of the 4 patients with evaluable samples for MRD, 3 patients (75.0%) achieved MRD-negative status (10^{-5} threshold) and also achieved sCR. One patient, initially MRD-negative on day 29, became MRD-positive on day 751, while the other 2 patients maintained MRD-negative status upon reassessment on days 710 and 716.[13]
- Median PFS and median OS were NE.[13]
- At the data cutoff, 5 patients (71.4%) were alive and without progression, including the patient who became MRD-positive on day 751. Additionally, the patient who was designated as NE for response remained alive and progression free.[13]

CARVYKTI® (ciltacabtagene autoleucel) CARTITUDE-1 study

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- All patients experienced grade ≥3 TEAEs, and no exacerbations of GVHD were observed.[13]
- Prolonged grade 3/4 thrombocytopenia (not recovered by day 30) occurred less frequently in alloSCT patients compared to non-alloSCT patients (0% vs 44.4%). However, recurrent lymphopenia was observed more often in patients with prior alloSCT vs the non-alloSCT patients (71.4% vs 27.8%).[13]
- All patients with prior alloSCT who had grade 3/4 thrombocytopenia (n=3) and lymphopenia (n=7) at baseline recovered to grade ≤2 by day 30.[13]
  - All patients with grade 3/4 neutropenia recovered to grade ≤2 by day 60. Grade 3/4 anemia recovered to grade ≤2 in 4 out of 5 patients. Overall, there was 1 recurrence each of grade 3/4 thrombocytopenia, neutropenia, and anemia beyond day 60, along with 5 recurrences of lymphopenia.
- CRS was reported in 6 patients with prior alloSCT (onset, 4-7 days post infusion); 3 were grade 1 and 3 were grade 2, and all resolved by 3-7 days. CRS treatment was given to 3 patients, which included tocilizumab (n=3), anakinra (n=1), corticosteroids (n=3), and intravenous fluids (n=3).[13]
- One patient with prior alloSCT experienced grade 1 ICANS.[13]
- Two deaths were reported in patients with prior alloSCT, both due to AEs (1 was considered treatment related).[13]
  - One patient developed unresolved MNTs/signs and symptoms of parkinsonism (maximum grade 3), including micrographia, masked facies, memory impairment, and somnolence, starting 15 days after CARVYKTI infusion.
  - This patient had established risk factors for MNTs/parkinsonism, including high tumor burden at baseline, and experienced grade 2 CRS and grade 1 ICANS. Eight days after infusion, ICANS with concurrent CRS occurred (both resolved within 3 days). The patient eventually developed pneumonia and died on day 119 due to a lung abscess with empyema, attributed to CARVYKTI.

Summary of TEAEs

Summary of TEAEs[13]

AEs, n (%)	Patients with prior alloSCT (n=7)
Any TEAE	7 (100)
Grade 1	0 (0)
Grade 2	0 (0)
Grade 3	2 (28.6)
Grade 4	4 (57.1)
Grade 5	1 (14.3)
CRS	6 (85.7)
Grade 3/4	0 (0)
Neurotoxicity	1 (14.3)
ICANS	1 (14.3)[a]
Other	1 (14.3)[b]
Deaths	2 (28.6)
Due to AE	2 (28.6)[c]
Treatment related	1 (14.3)

[a]Grade 1 ICANS.
[b]Grade 3 MNTs/parkinsonism.
[c]One patient died due to a lung abscess with empyema on day 119 (treatment related); the patient had recovered from bacterial sepsis on day 78 and was treated with piperacillin sodium/tazobactam sodium and received nasal canula low-flow oxygen for pneumonia (not treatment related) on day 88. The other patient died from refractory ascites from pre-existing noncirrhotic portal fibrosis and nonalcoholic steatosis on day 445 (not treatment related).

AE, adverse event; alloSCT, allogeneic stem cell transplant; CRS, cytokine release syndrome; ICANS, immune effector cell-associated neurotoxicity syndrome; MNT, movement and neurocognitive treatment-emergent adverse event; TEAE, treatment-emergent adverse event.

CARVYKTI® (ciltacabtagene autoleucel) CARTITUDE-1 study

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- For the 7 patients with prior alloSCT, the mean (SD) maximum CAR transgene concentration was 60,552 (26,145) copies/µg gDNA, achieved within a median of 11 days (range, 9-330). The transgene became undetectable at a median of 183 days (range, 77-246).[13]
- By day 100, serum sBCMA was no longer detectable in the majority of patients.[13]

Key PK parameters for CARVYKTI CAR transgene and CAR-positive T cells in the blood[13]

Parameter	Patients With Prior alloSCT (n=7)
CARVYKTI CAR transgene in the blood[a]	
C_{max}, copies/µg gDNA, mean (SD)	60,552 (26,145)
t_{max}, day, median (range)	11.2 (8.8-329.8)
t_{bql}, day, median (range)[b]	183.0 (76.8-246.2)
$t_{1/2}$, day, mean (SD)	NA
CAR-positive T cells in the blood	
C_{max}, cells/µL, mean (SD)	2009 (2324)
t_{max}, day, median (range)	12.9 (8.7-300.9)
t_{bql}, day, median (range)[a]	183.0 (76.8-246.2)
$t_{1/2}$, day, mean (SD)[a]	46.6 (52.1)

[a]For comparison, non-alloSCT patients (n=90) had mean (SD) Cmax of 47,770 (27,175), median T_{max} of 13 days, and median (range) t_{bql} of 101 days (28-981).
[b]n=3.

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ADA	Anti-drug antibody	**DOR**	Duration of response
AE	Adverse event	**ECOG PS**	Eastern Cooperative Oncology Group performance status
ALC	Absolute lymphocyte count	**E:T**	effector-to-target
alloSCT	Allogeneic stem cell transplant	**FLC**	Free Light Chain
ALT	Alanine aminotransferase	**Flu**	Fludarabine
AML	Acute myelogenous leukemia	**gDNA**	Genomic deoxyribonucleic acid
AST	Aspartate aminotransferase	**GVHD**	Graft-versus-host disease
ASTCT	American Society for Transplantation and Cellular Therapy	**HLH**	Hemophagocytic lymphohistiocytosis
autoSCT	Autologous stem cell transplant	**ICANS**	Immune effector cell-associated neurotoxicity syndrome
BCMA	B-cell maturation antigen	**IMWG**	International Myeloma Working Group
CAR	Chimeric antigen receptor	**IQR**	Interquartile range
CAR-T	Chimeric antigen receptor-T	**IRC**	Independent review committee
CD	Cluster of differentiation	**ISS**	International Staging System
CI	Confidence interval	**ITT**	Intention-to-treat
C_{max}	Maximum concentration	**LOT**	Lines of therapy
CMC	Chemistry, manufacturing, and controls	**MDS**	Myelodysplastic syndrome
CR	Complete response	**MM**	Multiple myeloma
CRS	Cytokine release syndrome	**MNT**	Movement and neurocognitive treatment-emergent adverse event
CSF	Cerebrospinal fluid	**mPFS**	Median progression-free survival
Cy	Cyclophosphamide	**MRD**	Minimal residual disease
DNA	Deoxyribonucleic acid	**NA**	Not assessable

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NCI-CTCAE	National Cancer Institute Common Terminology Criteria for Adverse Events	**RRMM**	Relapsed/refractory multiple myeloma
NE	Not estimable	**RP2D**	Recommended phase 2 dose
NR	Not reached	**sBCMA**	Soluble B-cell maturation antigen
ORR	Overall response rate	**sCR**	Stringent complete response
OS	Overall survival	**SD**	Standard deviation
PD	Pharmacodynamics	**SPM**	Second primary malignancy
PFS	Progression-free survival	**$t_{1/2}$**	Half-life
PI	Proteasome inhibitor	**t_{bql}**	Time at which concentrations were below the limit of quantification
PK	Pharmacokinetics	**TEAE**	Treatment-emergent adverse event
PR	Partial response	**t_{max}**	Time at which Cmax occurred
QC	Quality Check	**VGPR**	Very good partial response

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A literature search of MEDLINE®, Embase®, BIOSIS Previews®, Derwent Drug File databases (and/or other resources, including internal/external databases) was conducted on 19 May 2026.

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